UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **February 7, 2018**

AMICUS THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-33497**	**71-0869350**
(State or other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)
1 Cedar Brook Drive, Cranbury, NJ		**08512**
(Address of Principal Executive Offices)		(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(Former name or former address if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

Item 8.01. Other Events.

On February 7, 2018, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing additional positive data for the Company's Pompe Disease Phase 1/2 Study at the WORLD*Symposium*™ 2018 conference in San Diego, California. A copy of this press release is attached as Exhibit 99.1 while a copy of the presented data is attached as Exhibit 99.2.

In addition, the senior management of the Company is using the presentation attached as Exhibit 99.3 in its current meetings with investors and analysts.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

Exhibit No.	Description
99.1	Press Release dated February 7, 2018 titled "Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at 14th Annual WORLD*Symposium*™."
99.2	Poster — "Updated Results from ATB200-02: A First-in-Human, Open-Label, Phase 1/2 Study of ATB200 Coadministered With AT2221 in Adults With Pompe Disease."
99.3	Presentation Materials — "Amicus Pompe Overview & Pompe Data Highlights at 14th Annual WORLD*Symposium*™."

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AMICUS THERAPEUTICS, INC.

Date: February 7, 2018

By: /s/ ELLEN S. ROSENBERG

Name: Ellen S. Rosenberg

Title: General Counsel and Corporate Secretary

Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at 14th Annual WORLD*Symposium*™

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers Out to Month 12

Very Low Rate (<1%) of Infusion Associated Reactions Maintained After 550+ Infusions

CRANBURY, NJ, and San Diego, CA, February 7, 2018 — Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate ATB200/AT2221 in patients with Pompe disease, an inherited lysosomal storage disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells. Patients treated with ATB200/AT2221 for up to 12 months showed improvements in six-minute walk test (6MWT) distance and other measures of motor function, stability or increases in forced vital capacity (FVC), and durable reductions in biomarkers of muscle damage and disease substrate. These clinical results are being featured at the 14th Annual World*Symposium*™ in a late-breaker poster(1) today, and a corresponding oral platform presentation on Thursday, February 8, 2018 at 1:15pm PT.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, "We continue to be impressed by the clinical data for our novel Pompe treatment paradigm ATB200/AT2221. These latest data, in more patients and over longer periods, have continued to show substantial improvements in functional outcomes in nearly all patients, which are aligned with the persistent and durable reductions in key biomarkers of muscle damage and disease substrate. On the heels of these data for ATB200/AT2221 we look forward to continuing our discussions with global regulators to define the best and fastest pathway to deliver this critically important medicine to people living with Pompe disease."

Tahseen Mozaffar, MD, Director, Neuromuscular Program, Neurology School of Medicine at UC Irvine and Principal Investigator in the ATB200-02 study stated, "The results from this Phase 1/2 clinical study of ATB200/AT2221 show very meaningful improvements across functional measures in both ERT-switch and ERT-naïve patients for up to 12 months, with remarkable consistency across the vast majority of patients, as well as across endpoints. This treatment regimen has also been well tolerated by the patients in this study. Overall, the safety and functional data, in addition to the biomarkers of muscle damage and disease substrate, suggest the potential for ATB200/AT2221 to become an important treatment paradigm for people living with Pompe disease."

Amicus continues to engage in a series of collaborative discussions with regulators in the U.S. and EU, and expects to provide an update in the first half of 2018.

ATB200-02 Study Data Highlights in ERT-Switch and ERT-Naive Patients Out to Month 12

A copy of the *WORLDSymposium*™ poster and a slide deck summarizing the latest clinical results from the ATB200-02 clinical study is available at www.amicusrx.com.

Safety, Tolerability & Pharmacokinetics (PK) (n=20)

Safety and tolerability data in all 20 patients reflect a maximum of 20+ months of treatment. To date, adverse events have been generally mild and transient. Importantly, ATB200/AT2221 has resulted in a low rate of infusion-associated reactions (IARs) following 550+ infusions (three events of IARs in two patients; <1% of all 550+ infusions with an IAR). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Functional Outcomes (n=20)

Data on functional outcomes are available for 19 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations). Muscle function improved in 16 of 19 patients at month 9. Muscle function improved in 10 out of 10 patients with available data at month 12.

- **Motor function (n=15):** Six-minute walk test (6MWT) distance, a primary measure of motor function in Pompe disease patients, improved in both ERT-naive and ERT-switch patients with continued benefit observed out to month 12. Improvements were generally consistent across patients and cohorts. Additional detail on patient-level 6MWT distance data is available in the poster.
    - All 5 ERT-naive patients showed increases in 6MWT distance at all time points out to month 12. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 64 meters at month 9 (n=5), and 87 meters at month 12 (n=2).
    - Of the 10 ERT-switch patients, 8 patients showed increases in 6MWT distance and two patients showed decreases at month 9. All eight of the ERT-switch patients with available data at month 12 showed increases in 6MWT distance. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 25 meters at month 9 (n=10), and 57 meters at month 12 (n=8).
    - Other motor function tests, as detailed in the poster, generally showed mean improvements consistent with 6MWT distance.
- **Muscle Strength (n=4):** three of the four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (which includes elbow and shoulder) from baseline to month 9, as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT).
- **Pulmonary Function (n=14):** Pulmonary function improved in ERT-naïve patients and was generally stable in ERT-switch patients. In ERT-naïve patients, mean absolute change in forced vital capacity (FVC), one of the main measures of pulmonary function in Pompe disease, was +4.2% at month 6 (n=5), +6.2% at month 9 (n=5), and +6.0% at month 12 (n=2). In ERT-switch patients mean absolute change in FVC was -1.3% at month 6 (n=9), -1.7% at month 9 (n=9), and -3.1% at month 12 (n=7). Overall, other pulmonary tests of maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation, were stable or increased in both ERT-naïve and ERT-switch patients.

Pharmacodynamic (PD) Data on Muscle Damage and Disease Substrate Biomarkers (n=20)

Treatment with ATB200/AT2221 resulted in persistent and durable reductions in key biomarkers of muscle damage (creatine kinase, or CK) and disease substrate (urine hexose tetrasaccharide, or Hex4) across all patient cohorts out to month 12 and continue to suggest a positive effect on muscle tissue. Further details are provided in the poster.

About ATB200-02 Clinical Study

The primary objectives of the open-label ATB200-02 clinical study are to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 over an 18-week primary treatment period followed by a long-term extension. Sixteen clinical sites in five countries participated in the ATB200-02 clinical study. The study enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-switch (Cohort 1, n=11), non-ambulatory ERT-switch (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). Patients in Cohort 1 received escalating doses of ATB200 (5, 10, 20 mg/kg), followed by 3 doses of 20 mg/kg ATB200 plus low dose AT2221, followed by ongoing doses of 20 mg/kg ATB200 plus high dose AT2221. Patients in Cohorts 2 and 3 have all received 20 mg/kg ATB200 plus high dose AT2221.

About ATB200/AT2221

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced glycogen levels in muscle, which was further improved when AT2221 was co-administered with ATB200. Amicus Therapeutics is currently conducting a global Phase 1/2 study (ATB200-02) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of ATB200/AT2221.

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The lead program in the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with

---

additional approvals granted and pending in several geographies. The future value driver of the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

Forward-Looking Statements

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended September 30, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

(1) Mozaffar, *et. al.*, **14th Annual World*Symposium*™,** Updated results from ATB200-02: a first-in-human, open-label, phase 1/2 study of ATB200 co-administered with AT2221 in adults with Pompe disease

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Senior Director, Investor Relations
spellegrino@amicusrx.com
(609) 662-5044

Media:
Jennifer Paganelli
Media Relations
jpaganelli@purecommunications.com
(347) 658-8290

FOLD—G

LB-38

Updated Results From ATB200-02: A First-in-Human, Open-Label, Phase 1/2 Study of ATB200 Coadministered With AT2221 in Adults With Pompe

Mozaffar T,[1] Sitaraman S,[2] Barth JA,[2] Sathe S,[2] on behalf of the ATB200-02 Clinical Trial Investigators (Bratkovic D, Byrne BJ, Clemens P, Geberhiwot T, Kishnani P, Ming X, Schwenkreis P, Sivakumar K, van der Ploeg AT, Roberts M, Schoser B)

[1]University of California, Irvine, Orange, CA, USA; [2]Amicus Therapeutics, Inc., Cranbury, NJ, USA

INTRODUCTION

- Pompe disease is an inherited metabolic disease of impaired lysosomal glycogen clearance due to acid α-glucosidase (GAA) deficiency, which leads to accumulation of the substrate most prominently in the heart, skeletal muscle, and smooth muscle[1,2]
- Progressive accumulation of glycogen results in a spectrum of disease severity, often leading to organ failure and/or death. Skeletal muscle weakness and progressive respiratory involvement are predominant manifestations of late-onset Pompe disease (LOPD)[1,2]
- ATB200 is a next-generation recombinant human GAA (rhGAA) enzyme replacement therapy (ERT) designed with optimized glycosylation and high levels of mannose 6-phosphate residues for better uptake in target muscle tissues. The pharmacological chaperone AT2221 is coadministered with ATB200 to minimize denaturation of the enzyme in blood and maintain catalytic activity to deliver active ERT to lysosomes[3,4]

OBJECTIVE

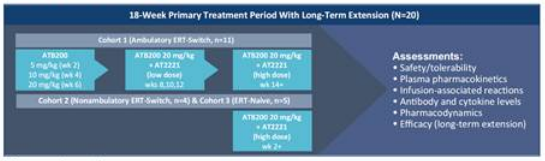
- To assess the safety, pharmacodynamics, and efficacy of ATB200 coadministered with AT2221 in patients with Pompe disease enrolled in the phase 1/2 ATB200-02 study (NCT02675465)

METHODS

Study Design

- Data are from interim analysis 5 and include all 12-month data that were available as of the data cutoff (n=8/11 for Cohort 1; n=1/4 for Cohort 2; n=2/5 for Cohort 3)
- Safety analyses include all data up to 20 months

Figure 1. ATB200-02 Study Design



ERT=enzyme replacement therapy.

Key Inclusion Criteria

- Males and females aged 18-65 years diagnosed with Pompe disease per documented deficiency of GAA enzyme activity or *GAA* genotyping
- Received ERT with alglucosidase alfa for 2-6 years (or ≥2 years for Cohort 2) prior to trial initiation (Cohort 1)
- Currently receiving alglucosidase alfa at a frequency of every other week and having completed the last 2 infusions without a drug-related adverse event (AE) resulting in dose interruption (Cohorts 1 and 2)
- Able to walk between 200 and 500 meters on the 6-Minute Walk Test (6MWT) (Cohorts 1 and 3)
- Upright forced vital capacity (FVC) 30-80% of predicted normal value (Cohorts 1 and 3)
- Wheelchair-bound and unable to walk unassisted (Cohort 2)

RESULTS

- Sixteen clinical sites in 5 countries participated in the ATB200-02 trial
- Patients were representative of the overall LOPD population, with significant impairment at baseline (**Table 1**)

Table 1. Baseline Characteristics

	Cohort 1 Ambulatory ERT-Switch N=11	Cohort 2 Nonambulatory ERT-Switch N=4	Cohort 3 ERT-Naive N=5
Age, years, mean (min, max)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex, M:F	9:2	3:1	1:4
Time on alglucosidase alfa, years, mean (SD)	4.8 (1.4)[a]	8.9 (3.8)	NA
6MWT, meters, mean (SD)	392.0 (93.4)	NA	399.5 (83.5)
Upright FVC, % predicted, mean (SD)	52.3 (13.2)	NA	53.4 (20.3)

6MWT=6-Minute Walk Test; FVC=forced vital capacity; NA=not applicable; SD=standard deviation.
[a]Cohort 1 patients were required to have been on alglucosidase alfa for 2-6 years at baseline.

Efficacy

- 6MWT improved for ambulatory ERT-switch patients and ERT-naive patients at Month 6 with continued benefit observed to Month 12 (**Table 2**)
  - 6MWT increased in 7/10, 8/10, and 8/8 ERT-switch patients at Months 6, 9, and 12, respectively
  - 6MWT increased in 5/5, 5/5, and 2/2 ERT-naive patients at Months 6, 9, and 12, respectively

Table 2. 6-Minute Walk Test, meters

Patient	Baseline	Change From Baseline		
		Month 6	Month 9	Month 12
Cohort 1 ERT-Switch				
1	544	+51	+56	+112
2	379	+125	+110	+103
3	339	+21	+45	+73
4	332	+8	+26	+45
5	456	−5	+8	+41
6	500	+55	+20	+33
7	220	+29	+21	+30
8	410	+38	+11	+22
9	464	−4	−9	—[a]
10	328	−78	−43	—[a]
Mean (SD)	397.2 (96.8)	+23.9 (52.2)	+24.5 (40.8)	+57.4 (34.4)
Cohort 3 ERT-Naive				
1	480	+41	+72	+95
2	384	+62	+78	+79
3	460	+79	+89	—[a]
4	406	+14	+44	—[a]
5	267	+13	+35	—[a]
Mean (SD)	399.5 (83.5)	+41.8 (29.4)	+63.5 (23.1)	+86.8 (11.1)

[a]12-month data not yet available.

- Improvements in 6MWT and other motor function tests were consistent with an overall improvement in motor performance for ambulatory ERT-switch patients and ERT-naive patients over 12 months (**Table 3**)

Table 3. Other Motor Function Tests

	Assessment, sec	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)	Change From Baseline to Month 9, Mean (SD)	Change From Baseline to Month 12, Mean (SD)
Cohort 1 ERT-Switch		n=10	n=10	n=10	n=8
	Timed Up and Go	10.5 (6.6)	−1.8 (3.5)	−1.2 (3.3)	−1.0 (2.2)
	4-Stair Climb	4.1 (2.7)	−0.6 (1.6)	−0.4 (1.6)	−1.0 (1.5)
	10M Walk	7.4 (3.0)	+0.1 (1.9)	−0.1 (1.6)	−0.5 (1.7)
	Gowers[a]	7.9 (2.9)	−1.1 (3.8)	+4.5[b] (13.4)	−2.6 (1.9)
	GSGC Score	12.6 (4.8)	+0.1 (3.9)	+0.5 (4.6)	−1.9 (2.2)
Cohort 3 ERT-Naive		n=5	n=5	n=5	n=2
	Timed Up and Go	9.4 (2.9)	−1.0 (1.1)	−0.6 (1.4)	−1.8 (0.5)
	4-Stair Climb	4.2 (1.5)	−0.6 (0.3)	0.0 (1.5)	−0.4 (0.4)
	10M Walk	7.9 (3.0)	−0.7 (1.1)	−1.3 (1.0)	−0.6 (0.0)
	Gowers	13.9 (11.0)	+7.9[c] (20.9)	−1.6 (3.9)	−2.1 (1.3)
	GSGC Score	12.2 (3.6)	−1.8 (3.8)	−2.4 (3.4)	0.0 (1.4)

GSGC=Gait, Stairs, Gowers, Chair.
GSGC is an observer-rated combined score of 4 motor function assessments: Gait (10m walk), 4-Stair Climb, Gowers (stand from floor), and Rising From Chair. Each test is scored from 1 (normal) to 7 (cannot perform; max score of 6 for Rising From Chair). Total scores range from 4 to 27.
[a]N=9, missing values not obtained due to patient refusal to perform test.
[b]Median change from baseline was −1.5, and 7/9 patients had a decrease.
[c]Median change from baseline was −0.8, and 4/5 patients had a decrease.

- Consistent and substantial increases were observed in upper extremity strength in nonambulatory ERT-switch patients at Month 6 and Month 9 (**Table 4**)
  - Three out of 4 patients showed improvements in upper extremity strength from baseline to Month 9 as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT)

Table 4. Muscle Strength Testing in Nonambulatory ERT-Switch Patients (cohort 2)

	Muscle Group Tested	Baseline, Mean (SD) n=4	Change From Baseline to Month 6, Mean (SD) n=4	Change from Baseline to Month 9, Mean (SD) n=4
Quantitative Muscle Testing—Dynamometer, pounds force	Shoulder Adduction[a]	5.7 (8.8)	+8.1 (12.8)	+9.6 (12.3)
	Shoulder Abduction	16.7 (18.1)	+1.0 (6.6)	+0.5 (9.3)
	Elbow Flexion	12.7 (13.7)	+2.4 (15.9)	+6.0 (19.3)
	Elbow Extension	12.3 (13.9)	+5.5 (4.7)	+7.5 (8.2)
Manual Muscle Testing, manual score[b]	Shoulder Adduction	2.3 (2.1)	+1.3 (2.3)	0.0 (4.0)
	Shoulder Abduction	2.7 (2.3)	+0.5 (0.7)	−1.0 (2.7)
	Elbow Flexion	4.3 (4.5)	+1.7 (1.5)	+1.7 (1.5)
	Elbow Extension	4.0 (4.0)	+1.7 (1.5)	+1.7 (1.5)

QMT results are pounds of force for right and left sides combined. MMT scores are for right and left sides combined. MMT scoring: 1) Visible muscle movement, but no movement at the joint; 2) Movement at the joint, but not against gravity; 3) Movement against gravity, but not against added resistance; 4) Movement against resistance, but less than normal; 5) Normal strength. Total MMT scores are out of 10 (right and left combined). Data shown to Month 9 because only 1 patient in Cohort 2 had Month 12 QMT and MMT data available at the time of the analysis.
[a]Shoulder adduction not available for 1 patient.
[b]n=3 due to assessment not being performed at some visits for some patients.

- FVC was generally stable in ambulatory ERT-switch patients and increased in ERT-naive patients (**Table 5**)
  - FVC was stable or increased in 5/9, 6/9, and 3/7 ERT-switch patients at Months 6, 9, and 12, respectively
  - FVC was stable or increased in 5/5, 5/5, and 2/2 ERT-naive patients at Months 6, 9, and 12, respectively
- Maximal inspiratory pressure (MIP) was stable and maximal expiratory pressure (MEP) increased in ambulatory ERT-switch patients; MIP increased and MEP was stable in ERT-naive patients (**Table 5**)

Table 5. Forced Vital Capacity and Other Pulmonary Function Tests

	Assessment	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)	Change From Baseline to Month 9, Mean (SD)	Change From Baseline to Month 12, Mean (SD)
Cohort 1 ERT-Switch		n=10	n=10	n=10	n=8
	FVC, % predicted[a]	52.6 (14.7)	−1.3 (4.1)	−1.7 (3.9)	−3.1 (4.8)
	MIP	35.7 (11.0)	+0.3 (4.6)	−0.6 (3.0)	+0.3 (3.6)
	MEP	72.6 (32.6)	+16.1 (42.1)	+23.7 (38.1)	+36.8 (45.7)
Cohort 3 ERT-Naive		n=5	n=5	n=5	n=2
	FVC, % predicted	53.4 (20.3)	+4.2 (5.6)	+6.2 (5.3)	+6.0 (7.1)
	MIP	32.6 (18.5)	+11.0 (5.0)	+12.0 (10.3)	−0.5 (9.2)
	MEP	60.6 (8.3)	−0.4 (12.4)	+7.2 (15.3)	−2.0 (9.9)

MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure.
[a]FVC not available for 1 patient.
MIP and MEP were measured in centimeters of water.

Patient-Reported Outcomes

- All cohorts were significantly impacted by fatigue at baseline, and dem their Fatigue Severity Scale (FSS) after receiving ATB200/AT2221 (**Table**

Table 6. Fatigue Severity Scale

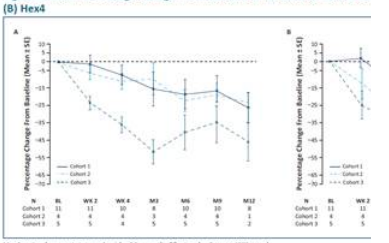
	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)
Cohort 1 Ambulatory ERT-Switch	n=10	n=10
	53.5 (7.7)	−8.0 (10.7)
Cohort 2 Nonambulatory ERT-Switch	n=4	n=2
	54.0 (8.5)	−3.5 (7.8)
Cohort 3 ERT-Naive	n=5	n=5
	39.2 (12.7)	−5.2 (11.7)

Fatigue Severity Scale consists of 9 questions, each scored on a scale from 1 to 7. The total score ranges fr higher levels of fatigue due to the disease condition. The normative value in the healthy population is ~21

Markers of Muscle Injury

- All cohorts demonstrated persistent improvement in biomarkers of mu and disease substrate (urine hexose tetrasaccharide; Hex4) for up to 12

Figure 2. Mean Percentage Change From Baseline in Markers of Muscle Da (B) Hex4



Hex4=urine hexose tetrasaccharide; M=month; SE=standard error; WK=week.

Safety

- At the data cutoff, the longest duration on treatment was 20+ months
- AEs were generally mild and transient
- The most common AEs reported as treatment related were upper and l diarrhea (8/20), nasopharyngitis (6/20), nausea (5/20), headache (5/20 infection (5/20)
- There were 3 incidents of infusion-associated reactions (IAR) in 550+ in standard premedication
  - One IAR in a nonambulatory ERT-switch patient (skin discoloratio
  - Two IARs in an ERT-naive patient (localized pruritus, erythema an

CONCLUSIONS

- Muscle function
  - 6MWT distance continued to improve in ambulatory ERT-switch Month 12
  - Other motor function tests were consistent with 6MWT results i
  - There were increases in elbow and shoulder muscle strength in n patients at Months 6 and Month 9
- Pulmonary function
  - FVC, MIP, and MEP were generally stable in ambulatory ERT-swit
  - FVC, MIP, and MEP generally increased in ERT-naive patients
- Fatigue Severity Scale
  - Improvements in fatigue score were observed in all cohorts
- Biomarkers and safety
  - CK and Hex4 levels decreased in all cohorts
  - ATB200/AT2221 was generally well tolerated

REFERENCES

1. Kishnani PS et al. *Genet Med*. 2006;8(5):267-288.
2. Bijvoet AGA et al. *Hum Mol Gen*. 1998;7(1):53-62.
3. Gotschall R et al. *Mol Genet Metab*. 2015;114(2):549.
4. Khanna R et al. Presented at the 12th Annual WORLDSymposium™; February 29-Ma
5. Grace J et al. *Parkinsonism Relat Disord*. 2007;13:443-445.

ACKNOWLEDGMENTS

The authors thank the patients, their families, and Pompe disease patient o investigators. Third-party medical writing assistance was provided by Apoth supported by Amicus Therapeutics, Inc.

DISCLOSURES

Conflicts of Interest

T. Mozaffar has served as a consultant for Amicus Therapeutics and Sanofi G member of a speaker bureau for Sanofi Genzyme. S. Sitaraman, J.A. Barth, a employees of and own stock in Amicus Therapeutics.

Supported by Amicus Therapeutics, Inc.

Presented at the 14th Annual WORLD*Symposium*™; Febr

Amicus Therapeutics

Amicus Pompe Overview & Pompe Data Highlights at 14th Annual World*Symposium*™

February 5-9, 2018 | San Diego, CA

Pompe Disease Overview

Dr. Priya Kishnani

14th Annual WORLDSymposium™ | February 5-9, 2018 | San Diego, CA

Disclosure Information

WORLD*Symposium*™ 2018 | *Dr. Priya Kishnani*

I have the following financial relationships to disclose:
Grant/Research support from: Genzyme
Honoraria from: Amicus Therapeutics, Genzyme

- and -

I will not discuss an off label use and/or investigational use in my presentation.

Our Team

- Over 70 members at Duke in Pompe disease clinical care/research
- Follow close to 250 Pompe patients at Duke
- Follow another 150-200 patients globally
- Provided care to patients from around the world- in person, telemedicine, email, phone
- Helped with drug approval in several countries including Singapore, Malaysia, Australia, Latin America, etc.
- Facilitated discussions with FDA and European Union for approval in US and EMEA
- Global outreach via charitable access programs in several countries including India, South Africa, China, Egypt, Israel, Peru, Brazil, Argentina, Chile, etc

Duke Contribution to the Field

Pompe Disease

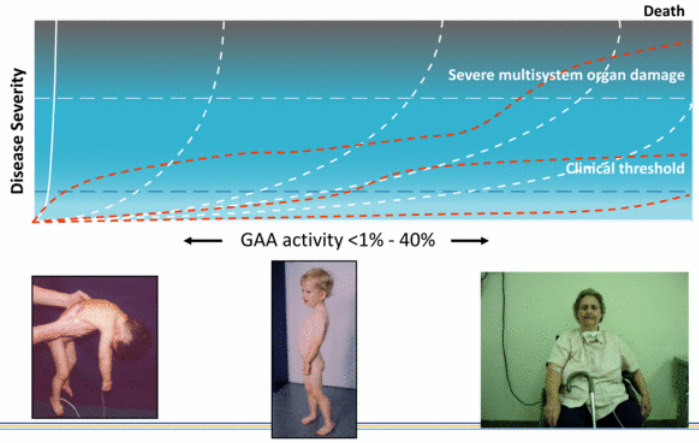
- Metabolic myopathy characterized by cardiac, skeletal and smooth muscle involvement with a continuum of disease severity
  - From early onset → rapid progression to death (infantile onset)
  - To later onset → slower progression, longer survival with marked morbidity (late onset)
- Deficiency of lysosomal enzyme, acid alpha-glucosidase (GAA)
- Glycogen accumulation → muscle tissue damage → functional impairment → permanent disability
- Variable rate of tissue damage in muscle

Pompe Disease

A continuum of disease caused by deficiency of acid alfa glucosidase (GAA)

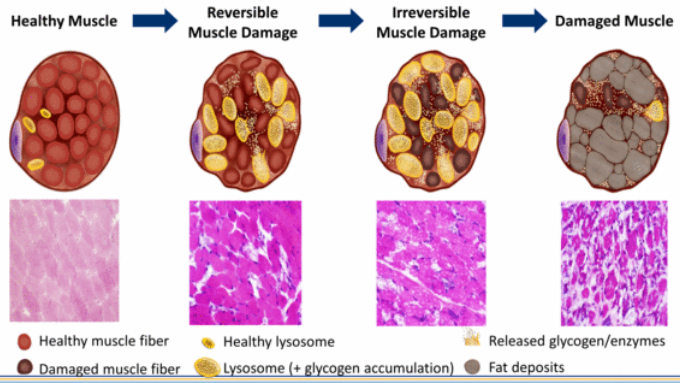
- Infantile Onset Pompe Disease (IOPD)
  - Presents in the *first few days* to months with hypotonia, generalized muscle weakness, macroglossia
  - Hypertrophic cardiomyopathy leads to death within the first year
- Late Onset (Juvenile and Adult) Pompe Disease (LOPD)
  - Characterized by respiratory and limb-girdle muscle weakness, resulting in significant morbidity and mortality
  - Lack of *severe* cardiac involvement
  - Early involvement of the diaphragm

Pompe Disease: A Continuum of Clinical Phenotypes



The Natural Course of Pompe Disease is a Progression From Healthy Muscle to Irreversible Muscle Damage

Untreated Pompe Disease



Courtesy of Dr. Priya Kishnani; Kishnani PR, et al. Am J Med Genet C Semin Med Genet. 2012;160C:1-7; Kishnani PR, et al. Genet Med. 2006;8:267-88.

Current Standard of Care and Factors Affecting Response to ERT

- Multidisciplinary
- Enzyme replacement therapy (ERT) with recombinant human GAA at 20 mg/kg every 2 weeks
- Factors Affecting Response to ERT
  - Degree of overall muscle damage and extent of preexisting pathology[2]
  - Age/Disease duration upon ERT initiation[2]
  - Predominance of Muscle fiber type (*i.e.,* type I *vs.* type II)[1]
  - Degree of disordered cellular processes, such as defective autophagy[1]
  - ACE polymorphism (D/D phenotype a poor prognostic factor)[4]
  - Cross-reactive immunologic material (CRIM) negative status[2]
  - Degree of any immunological reaction to therapy (high sustained titers and persistent titers)[3]

1. Raben, N et al. Enzyme replacement therapy in the mouse model of Pompe disease. Mol Genet Metab, 2003.
2. Kishnani, PS et al. Cross-reactive immunologic material status affects treatment outcomes in Pompe disease infants. Mol Genet Metab, 2010.
3. Banugaria, S et al. Persistence of high sustained antibodies to enzyme replacement therapy despite extensive immunomodulatory therapy in an infant with Pompe disease: need for agents to target antibody-secreting plasma cells. Mol Genet Metab, 2012.
4. P. De Fillipi et al. The angiotensin-converting enzyme insertion/deletion polymorphism modifies the clinical outcome in patients with Pompe disease

Long-term Issues and Emerging Phenotype in IOPD (Clinically Diagnosed and Treated)

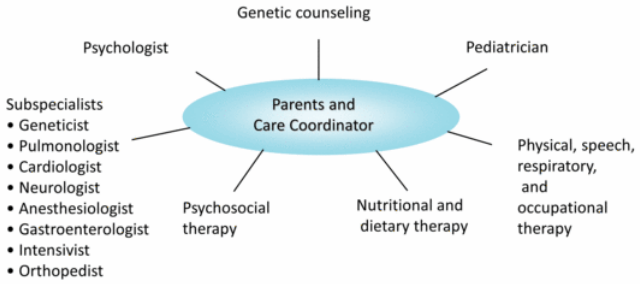
- **Cardiac**
  - Fibrosis
  - Cardiac arrhythmias
  - Dilatation of aorta
- **Neurologic**
  - Sensorineural hearing loss
  - Anterior horn cell involvement
  - Bulbar involvement
  - White matter changes, questions related to cognition
- **Speech acquisition**
  - Hypernasal speech due to velopharyngeal weakness and facial weakness
- **Ophthalmologic findings**
  - Ptosis-myogenic
  - Severe myopia
- **Sphincter issues**

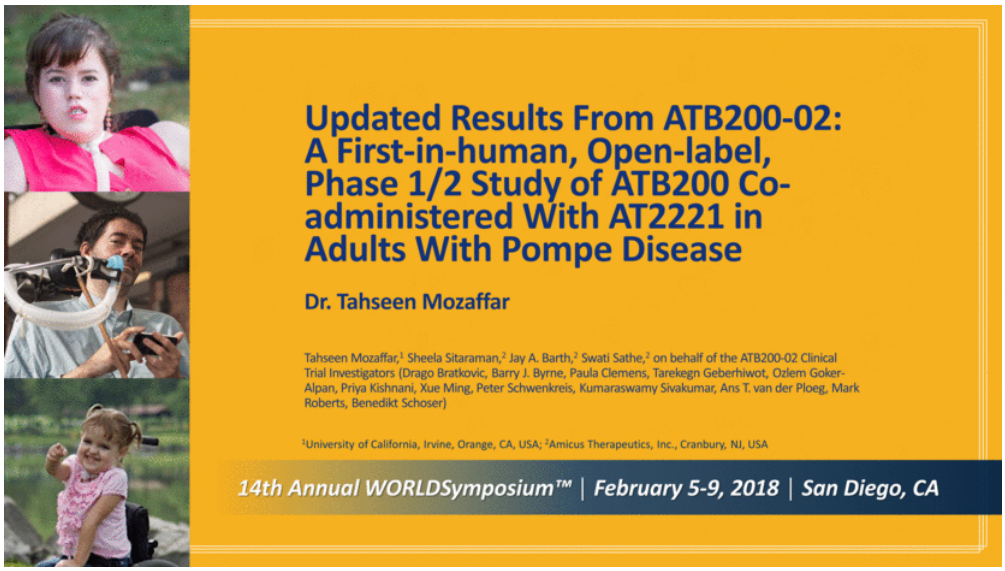
Prater SN, Banugaria SG, et al. The Emerging Phenotype of Long-Term Survivors with Infantile Pompe Disease. *Genetics in Medicine.* 2012; 14: 800-810.

Issues in Late Onset Pompe Disease

- Diagnosed late, very clinically heterogenous
- Response to current ERT typically noted in first 12-18 months, then a stabilization/decline
- Inefficient targeting of current ERT especially in skeletal muscle (poor M6P receptor in skeletal muscle)
- Immune response can occur
- Long duration of infusion

Pompe Disease Management Requires Coordination of Multi-Disciplinary Care





Updated Results From ATB200-02: A First-in-human, Open-label, Phase 1/2 Study of ATB200 Co-administered With AT2221 in Adults With Pompe Disease

Dr. Tahseen Mozaffar

Tahseen Mozaffar,[1] Sheela Sitaraman,[2] Jay A. Barth,[2] Swati Sathe,[2] on behalf of the ATB200-02 Clinical Trial Investigators (Drago Bratkovic, Barry J. Byrne, Paula Clemens, Tarekegn Geberhiwot, Ozlem Goker-Alpan, Priya Kishnani, Xue Ming, Peter Schwenkreis, Kumaraswamy Sivakumar, Ans T. van der Ploeg, Mark Roberts, Benedikt Schoser)

[1]University of California, Irvine, Orange, CA, USA; [2]Amicus Therapeutics, Inc., Cranbury, NJ, USA

14th Annual WORLDSymposium™ | February 5-9, 2018 | San Diego, CA

Disclosure Information

WORLD*Symposium*™ 2018 | *Dr. Tahseen Mozaffar*

I have the following financial relationships to disclose:

- Consultant for Amicus Therapeutics, Inc.
- Consultant and member of speaker bureau for Genzyme

I will discuss the following off-label use and/or investigational use in my presentation:

- Data from a phase 1/2 trial of ATB200/AT2221 in patients with Pompe disease
- ATB200 and AT2221 are investigational drugs that have not been approved for use in the United States

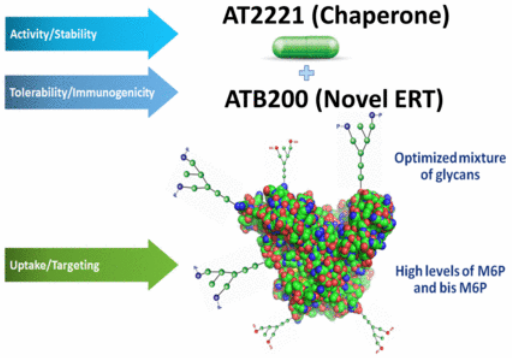
Overview of Novel Pompe Approach ATB200/AT2221

- Amicus Therapeutics is developing a combination therapeutic approach with two investigational agents:
  - Oral administration of pharmacological chaperone (PC), AT2221[1], prior to
  - IV infusion of ATB200 (rhGAA) enzyme replacement therapy (ERT)

GAA=acid α-glucosidase; rhGAA=recombinant human acid α-glucosidase.
1. Kishnani PS et al. *Genet Med.* 2006;8(5):267-288. 2. Bijvoet AGA et al. *Hum Mol Gen.* 1998;7(1):53-62.

ATB200 Co-administration With AT2221

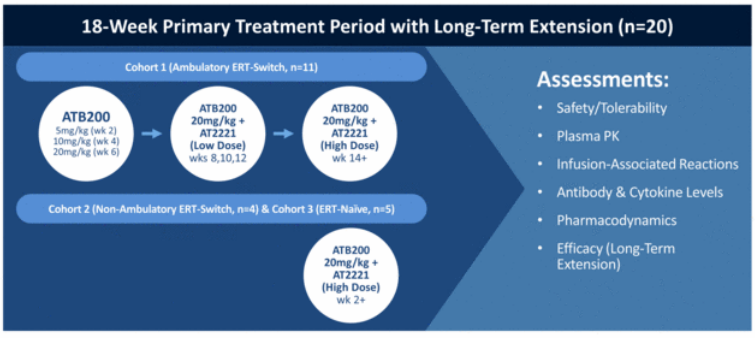
- AT2221: orally administered investigational PC prior to infusion of ATB200
  - Shown to stabilize ERT in blood and maintain catalytic activity to enhance delivery of active enzyme to lysosomes[1,2]
- ATB200: investigational next-generation ERT
  - Designed with optimized glycosylation and high levels of mannose 6-phosphate residues for better uptake to target tissues



ERT=enzyme replacement therapy; M6P=mannose-6-phosphate; PC=pharmacological chaperone
1. Gotschall R et al. *Mol Genet Metab.* 2015;114(2):S49. 2. Khanna R et al. Presented at the 12th Annual WORLDSymposium™; February 29-March 4, 2016; San Diego, CA, USA

ATB200-02 Study Design (NCT02675465)

Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221) at 16 Sites in 5 Countries



Baseline Characteristics (N=20)

Patients Enrolled Across Three Cohorts are Representative of the Overall Late-Onset Pompe Population, with Significant Impairment at Baseline

	Cohort 1 ERT-Switch (N=11)	Cohort 2 ERT-Switch Non-ambulatory (N=4)	Cohort 3 ERT-Naïve (N=5)
Age, years, mean (min, max)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex, M:F	9:2	3:1	1:4
Time on alglucosidase alfa, years, mean (SD)	4.8 (1.42)[a]	8.9 (3.8)	-
6MWT, meters, mean (SD)	392.0 (93.4)	NA	399.5 (83.5)
FVC Upright, % predicted, mean (SD)	52.3 (13.2)	NA	53.4 (20.3)

NA=not applicable; SD=standard deviation.
[a]Cohort 1 patients were required to have been on alglucosidase alfa for 2-6 years at baseline.

6-Minute Walk Test (6MWT) (n=15)

6MWT Improved for Both ERT-Naïve and ERT-Switch Patients at Months 6 and 9 With Continued Benefit Observed Out to Month 12

6-Minute Walk Test (m); mean (SD)

	Baseline (n=10)	Change to Month 6 (n=10)	Change to Month 9 (n=10)	Change to Month 12 (n=8)
Cohort 1 ERT-Switch	397.2 (96.8)	+23.9 (52.2)	+24.5 (40.8)	+57.4 (34.4)
	Baseline (n=5)	**Change to Month 6 (n=5)**	**Change to Month 9 (n=5)**	**Change to Month 12 (n=2)**
Cohort 3 ERT-Naïve	399.5 (83.5)	+41.8 (29.4)	+63.5 (23.1)	+86.8 (11.1)

- 6MWT increased in 7/10, 8/10, and 8/8 ERT-switch patients at Months 6, 9 and 12 respectively
- 6MWT increased in 5/5, 5/5, and 2/2 ERT-naïve patients at Months 6, 9 and 12 respectively

CFBL=change from baseline.

6-Minute Walk Test Patient-Level Data – Cohort 1 ERT-Switch (n=10)

6MWT Improved for ERT-Switch Patients at Months 6 and 9 With Continued Benefit Observed Out to Month 12

6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 9	Month 12
1052	544	+51	+56	+112
1252	379	+125	+110	+103
1251	339	+21	+45	+73
1751	332	+8	+26	+45
1201	456	-5	+8	+41
1451	500	+55	+20	+33
1051	220	+29	+21	+30
1053	410	+38	+11	+22
1701	464	-4	-9	N/A
1601	328	-78	-43	N/A
Mean (SD)	397.2 (96.8)	+23.9 (52.2)	+24.5 (40.8)	+57.4 (34.4)

- 6MWT increased in 7/10, 8/10, and 8/8 ERT-switch patients at Months 6, 9 and 12 respectively

N/A = data not available (patients have not reached 12 month time point)

6-Minute Walk Test Patient Level Data – Cohort 3 ERT-Naïve (n=5)

All Five ERT-Naive Patients Showed Increases in 6MWT Distance Out to Month 12

6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 9	Month 12
3551	480	+41	+72	+95
3552	384	+62	+78	+79
3051	460	+79	+89	N/A
3554	406	+14	+44	N/A
3553	267	+13	+35	N/A
Mean (SD)	**399.5 (83.5)**	**+41.8 (29.4)**	**+63.5 (23.1)**	**+86.8 (11.1)**

- 6MWT increased in 5/5, 5/5, and 2/2 ERT-naïve patients at Months 6, 9 and 12 respectively

N/A = data not available (patients have not reached 12 month time point)

Other Motor Function Tests (n=15)

Improvement in Other Motor Function Tests Is Consistent with an Overall Improvement in Motor Performance for Both ERT-Switch and ERT-Naïve Patients over 12 Months

Cohort	Assessment (sec)	Baseline Mean (SD), n=10	Change to Month 6 Mean (SD), n=10	Change to Month 9 Mean (SD), n=10	Change to Month 12 Mean (SD), n=8
Cohort 1: ERT-Switch	Timed up and Go	10.5 (6.6)	-1.8 (3.5)	-1.2 (3.3)	-1.0 (2.2)
	4 Stair Climb	4.1 (2.7)	-0.6 (1.6)	-0.4 (1.6)	-1.0 (1.5)
	10M walk	7.4 (3.0)	+0.1 (1.9)	-0.1 (1.6)	-0.5 (1.7)
	Gowers[a]	7.9 (2.9)	-1.1 (3.8)	4.5[b] (13.4)	-2.6 (1.9)
	GSGC Score	12.6 (4.8)	+0.1 (3.9)	+0.5 (4.6)	-1.9 (2.2)
Cohort	**Assessment (sec)**	**Baseline Mean (SD), n=5**	**Change to Month 6 Mean (SD), n=5**	**Change to Month 9 Mean (SD), n=5**	**Change to Month 12 Mean (SD), n=2**
Cohort 3: ERT-Naïve	Timed up and Go	9.4 (2.9)	-1.0 (1.1)	-0.6 (1.4)	-1.8 (0.5)
	4 Stair Climb	4.2 (1.5)	-0.6 (0.3)	0.0 (1.5)	-0.4 (0.4)
	10M walk	7.9 (3.0)	-0.7 (1.1)	-1.3 (1.0)	-0.6 (0.0)
	Gowers	13.9 (11.0)	7.9[c] (20.9)	-1.6 (3.9)	-2.1 (1.3)
	GSGC Score	12.2 (3.6)	-1.8 (3.8)	-2.4 (3.4)	0.0 (1.4)

[a]N=9 Missing values not obtained due to patient refusal to perform test; [b] Median CFBL was -1.5 and 7/9 had decrease [c] Median CFBL was -0.8 and 4/5 had decrease

Muscle Strength Testing (QMT), Manual Muscle Testing (MMT): Cohort 2

Substantial Increases Observed in Upper Extremity Strength in Non-Ambulatory ERT-Switch Patients at Month 6 and Month 9

Assessment	Muscle Group Tested	Baseline (n=4)	Change to Month 6 (n=4)	Change to Month 9 (n=4)
QMT- Quantitative Muscle Testing - Dynamometer (pounds force)	Shoulder Adduction *	5.7 (8.8)	+8.1 (12.8)	+9.6 (12.3)
	Shoulder Abduction	16.7 (18.1)	+1.0 (6.6)	+0.5 (9.3)
	Elbow Flex	12.7 (13.7)	+2.4 (15.9)	+6.0 (19.3)
	Elbow Extension	12.3 (13.9)	+5.5 (4.7)	+7.5 (8.2)
Assessment	**Muscle Group Tested**	**Baseline ** (n=3)**	**Change to Month 6 (n=3)**	**Change to Month 9 (n=3)**
MMT - Manual Muscle Testing (manual score)	Shoulder Adduction	2.3 (2.1)	+1.3 (2.3)	0.0 (4.0)
	Shoulder Abduction	2.7 (2.3)	+0.5 (0.7)	-1.0 (2.7)
	Elbow Flex	4.3 (4.5)	+1.7 (1.5)	+1.7 (1.5)
	Elbow Extension	4.0 (4.0)	+1.7 (1.5)	+1.7 (1.5)

CFBL=change from baseline.; * *Shoulder adduction not available for one subject;* ** Total Score MMT = 10 (R+L) N=3 due to assessment not being performed at some visits for some patients

Forced Vital Capacity (FVC) Summary (n=14)

FVC Increased In ERT-Naïve Patients and was Generally Stable in ERT-Switch Patients

FVC (% Predicted); mean (SD)

Cohort 1 ERT-Switch*	**Baseline (n=9)**	**Change to Month 6 (n=9)**	**Change to Month 9 (n=9)**	**Change to Month 12 (n=7)**
	52.6 (14.7)	**-1.3** (4.1)	**-1.7** (3.9)	**-3.1** (4.8)
Cohort 3 ERT-Naïve	**Baseline (n=5)**	**CFBL M6 (n=5)**	**CFBL M9 (n=5)**	**CFBL M12 (n=2)**
	53.4 (20.3)	**+4.2** (5.6)	**+6.2** (5.3)	**+6.0** (7.1)

- FVC was stable or increased in 5/9, 6/9, and 3/7 ERT-switch patients at Months 6, 9 and 12 respectively
- FVC was stable or increased in 5/5, 5/5, and 2/2 ERT-naïve patients at Months 6, 9 and 12 respectively

CFBL=change from baseline.; *FVC not available for one subject

Amicus Therapeutics

Other Pulmonary Function Tests: MIP and MEP (n=15)

Overall MIP and MEP were Stable or Increased in Both ERT-Naïve and ERT-Switch Patients

MIP and MEP; mean (SD)

Patients	Assessment	Baseline (n=10)	Change to Month 6 (n=10)	Change to Month 9 (n=10)	Change to Month 12 (n=8)
Cohort 1: ERT-Switch	**MIP**	**35.7** (11.0)	**+0.3** (4.6)	**-0.6** (3.0)	**+0.3** (3.6)
	MEP	**72.6** (32.6)	**+16.1** (42.1)	**+23.7** (38.1)	**+36.8** (45.7)
Patients	**Assessment**	**Baseline (n=5)**	**Change to Month 6 (n=5)**	**Change to Month 6 (n=5)**	**Change to Month 12 (n=2)**
Cohort 3: ERT-Naïve	**MIP**	**32.6** (18.5)	**+11.0** (5.0)	**+12.0** (10.3)	**-0.5** (9.2)
	MEP	**60.6** (8.3)	**-0.4** (12.4)	**+7.2** (15.3)	**-2.0** (9.9)

CFBL=change from baseline. MIP & MEP measured in cmH_2O ; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure.

Amicus Therapeutics

Fatigue Severity Scale (FSS) (n=19)

All Cohorts Were Significantly Impacted By Fatigue at Baseline and Demonstrated a Mean Improvement After Receiving ATB200/AT2221

Fatigue Severity Scale; mean score (SD)

Cohort 1: ERT-Switch	**Baseline (n=10)**	**Change to Month 6 (n=10)**	**Change to Month 9 (n=10)**	**Change to Month 12 (n=8)**
	53.5 (7.7)	**-8.0** (10.7)	**-6.8** (6.8)	**-7.8** (6.0)
Cohort 2: Non-Ambulatory ERT-Switch	**Baseline (n=4)**	**Change to Month 6 (n=2)**	**Change to Month 9 (n=2)**	**N/A**
	54.0 (8.5)	**-3.5** (7.8)	**-6.5** (5.0)	N/A
Cohort 3: Naïve	**Baseline (n=5)**	**Change to Month 6 (n=5)**	**Change to Month 9 (n=5)**	**Change to Month 12 (n=2)**
	39.2 (12.7)	**-5.2** (11.7)	**-7.8** (7.5)	**-1.5** (2.1)

Fatigue Severity Scale (FSS) consists of 9 questions, each scored on a scale from 1-7. The total score ranges from 9 to 63, with higher values representing higher level of fatigue due to the disease condition. The normative value in healthy population is ~21 [1].

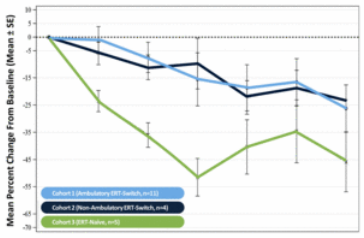
[1] Grace J et al. *Parkinsonism Relat Disord.* 2007;13(7):442-445.

Amicus Therapeutics

CK and Hex4 Biomarkers (n=20)

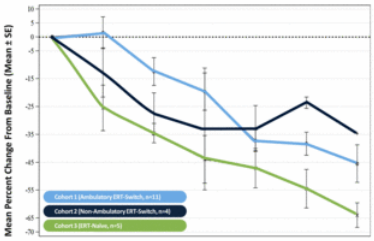
All Cohorts Demonstrated Persistent Improvement in Biomarkers of Muscle Damage (CK) and Disease Substrate (Hex4) For Up To 12 Months

Percent Change from Baseline for CK



Visit	BL	WK 2	WK 4	M3	M6	M9	M12
N	11,4,5	11,4,5	10,4,4	8,3,5	10,4,5	10,4,5	8,1,2

Percent Change from Baseline for Hex 4



Visit	BL	WK 2	WK 4	M3	M6	M9	M12
N	11,4,5	11,4,5	11,4,5	11,4,5	10,4,5	9,4,5	7,1,2

CK=creatine kinase; Hex4=urine hexose tetrasaccharide.
Missing values either unable to be analyzed or not yet analyzed.

Safety Summary (n=20)*

Safety Data for ATB200/AT2221 Show AEs Have Been Generally Mild and Transient with Very Low Rate of Infusion-Associated Reactions (< 1%) After 550⁺ Total Infusions Across All Cohorts

- AEs were generally mild and transient
  - Most common treatment emergent AEs (TEAEs) were abdominal pain** (8/20), diarrhea (8/20), nasopharyngitis (6/20), nausea (5/20), headache (5/20), upper respiratory tract infection (5/20).
- Three incidents of infusion-associated reactions in 550⁺ infusions which were controlled by standard premedication
  - One IAR event in one non-ambulatory ERT-switch patient (skin discoloration)
  - Two IAR events in one ERT-naïve patient (localized pruritus, erythema and burning sensation)
- Longest duration of treatment is 20⁺ months

AE, adverse events; IAR, infusion-association reaction.
*Reported through interim data analysis (maximum 20⁺ months)
**Includes upper and lower abdominal pain

Conclusions

- Muscle function
  - 6MWT distance generally improved in ERT-switch ambulatory and ERT-naïve patients out to month 12
  - Other motor function tests generally consistent with 6MWT results in both cohorts
  - Increases in elbow and shoulder muscle strength in non-ambulatory ERT-switch patients at Months 6 and 9
- Pulmonary function
  - FVC, MIP and MEP were generally stable in ERT-switch patients
  - FVC, MIP and MEP generally increased in ERT-naïve patients
- Fatigue Severity Scale
  - Improvement in fatigue score observed in all cohorts
- Biomarkers and Safety
  - CK and Hex4 levels decreased in all cohorts
  - ATB200/AT2221 was generally well tolerated, with low rate of infusion reactions

Data on file.

Acknowledgments

- The authors thank the patients, their families, and Pompe disease patient organizations, as well as the study investigators
- Third-party medical editing assistance was provided by ApotheCom and was supported by Amicus Therapeutics, Inc.

Amicus Therapeutics

Thank You

Amicus Therapeutics